Verve Therapeutics Announces Pipeline Progress and Reports Fourth Quarter and Full Year 2024 Financial Results

February 27, 2025 12:00 PM EST

Initial data from the Heart-2 Phase 1b clinical trial of VERVE-102 targeting PCSK9 expected in the second quarter of 2025, with final dose escalation data in the second half of 2025

VERVE-102 has been well-tolerated, with no treatment-related serious adverse events and no clinically significant laboratory abnormalities observed

Delivery of opt-in package to Eli Lilly and an opt-in decision from Lilly expected in the second half of 2025

Enrollment ongoing in Pulse-1 Phase 1b clinical trial of VERVE-201 targeting ANGPTL3

Cash, cash equivalents, and marketable securities of $524.3 million; cash runway into mid-2027

BOSTON, Feb. 27, 2025 (GLOBE NEWSWIRE) -- Verve Therapeutics, a clinical-stage company developing a new class of genetic medicines for cardiovascular disease, today reported pipeline and corporate updates and financial results for the fourth quarter and year ended December 31, 2024.

“2024 was a year of strong execution as we made crucial progress across our pipeline and laid the groundwork for 2025 to be a milestone-rich year for Verve,” said Sekar Kathiresan, M.D., co-founder and chief executive officer of Verve Therapeutics. “Enrollment for the Heart-2 Phase 1b clinical trial of VERVE-102 is progressing well, and dosing in the third cohort continues. As of February 13, 2025, VERVE-102 continues to be well-tolerated, and we are on track to report initial data from the Heart-2 trial in the second quarter of this year. We anticipate additional milestones in the second half of 2025, including final data from the dose escalation portion of the Heart-2 trial, delivery of the opt-in package and an opt-in decision from Lilly on the PCSK9 program, as well as a program update for VERVE-201, our program targeting *ANGPTL3*.”

Dr. Kathiresan continued, “As we capitalize on this tremendous momentum, we want to extend our deep gratitude to our chief medical officer, Dr. Frederick Fiedorek, who has decided to retire. Fred has set up Verve’s clinical programs for success and will continue to serve in an advisory capacity as Scott Vafai, M.D., Senior Vice President, Clinical Development, assumes responsibility for our clinical team. Supported by a strong balance sheet, we are excited about the opportunities ahead as we advance our pipeline of one-time treatments that have the potential to offer a lifetime of benefit for cardiovascular disease patients.”

PCSK9 Program
Initial Data for the Heart-2 Phase 1b Clinical Trial Evaluating VERVE-102 Expected in the Second Quarter of 2025

- VERVE-102 is a novel, *in vivo,* investigational base editing medicine designed to be a single course treatment that permanently turns off the *PCSK9* gene in the liver and durably reduces disease-driving low-density lipoprotein cholesterol (LDL-C). VERVE-102 uses Verve’s proprietary GalNAc lipid nanoparticle (LNP) delivery technology, which is designed to allow the LNP to access liver cells using either the low-density lipoprotein receptor (LDLR) or the asialoglycoprotein receptor (ASGPR).
- VERVE-102 is being evaluated in the Heart-2 open-label Phase 1b clinical trial in two patient populations who require deep and durable reductions of LDL-C levels in the blood: adults living with heterozygous familial hypercholesterolemia (HeFH) and adults living with premature coronary artery disease (CAD). The Heart-2 clinical trial is expected to include four dose cohorts, each comprised of three to nine participants with either HeFH or premature CAD.
- Dosing has been completed or is ongoing in participants across the first three dose cohorts, 0.3 mg/kg, 0.45 mg/kg, and 0.6 mg/kg, in the Heart-2 clinical trial. As of the data cut-off date of February 13, 2025, VERVE-102 has been well-tolerated, with no treatment-related serious adverse events and no clinically significant laboratory abnormalities observed.
- Verve expects to announce demographic and initial safety and efficacy data from the Heart-2 clinical trial as well as an update on the PCSK9 program in the second quarter of 2025. Enrollment is progressing well, and this initial data set is expected to include participants across the first three dose cohorts (0.3 mg/kg, 0.45 mg/kg, and 0.6 mg/kg) with at least 28 days of follow-up for each participant.
- Verve expects to report the final data for the dose escalation portion of the Heart-2 clinical trial and initiate the Phase 2 clinical trial for the PCSK9 program in the second half of 2025.
- Verve plans to deliver the opt-in data package for the PCSK9 program and receive a decision from Eli Lilly and Company (Lilly) in the second half of 2025.

ANGPTL3 Program
Pulse-1 Phase 1b Clinical Trial for VERVE-201 Continues to Progress

- VERVE-201 is a novel, *in vivo*, investigational base editing medicine designed to be a single course treatment that permanently turns off the *ANGPTL3* gene in the liver to reduce disease-driving LDL-C as well as remnant cholesterol and utilizes Verve’s proprietary GalNAc-LNP delivery technology.
- VERVE-201 is being developed in two patient populations: patients with refractory hypercholesterolemia (RH), defined as those who are unable to achieve adequate LDL-C reduction with maximally tolerated standard of care therapies, potentially including PCSK9 inhibitors, and patients living with homozygous familial hypercholesterolemia (HoFH), a rare and often fatal inherited cause of premature atherosclerotic cardiovascular disease (ASCVD) characterized by extremely high blood LDL-C. The aim of this medicine is to reduce the heavy treatment burden associated with available therapies, including the requirement for multiple oral, injectable, and intravenous infusions, often administered over decades.
- In November 2024, Verve announced that the first participant was dosed with VERVE-201 in its Pulse-1 open-label Phase 1b clinical trial. The Pulse-1 clinical trial is designed to evaluate the safety and tolerability of VERVE-201 administration in adult patients with RH who require

additional lowering of LDL-C despite treatment with maximally tolerated standard of care therapies, potentially including PCSK9 inhibitors. Endpoints also include pharmacokinetics and changes in blood ANGPTL3 protein and LDL-C levels.
- Verve expects to provide an update on the ANGPTL3 program in the second half of 2025.

LPA Program
Development for VERVE-301 Ongoing

- In January 2025, Verve announced the nomination of VERVE-301 as the company's development candidate targeting the *LPA* gene. VERVE-301 uses a novel, *in vivo* gene editing approach designed to permanently turn off the *LPA* gene in the liver to reduce blood lipoprotein (a) [Lp(a)] levels.
- Lp(a) is a genetically validated, independent risk factor for ASCVD, ischemic stroke, thrombosis, and aortic stenosis. This increased risk is most pronounced in individuals with very high Lp(a) concentrations (e.g., ≥ 125 nmol/L). An estimated 1.4 billion people worldwide have an Lp(a) concentration above this threshold. Lp(a) concentrations are determined at birth. Unfortunately, lifestyle changes - such as diet and exercise - as well as currently approved lipid-lowering therapies have minimal to no impact on reducing Lp(a) levels.
- Verve has an exclusive research collaboration with Lilly to advance its *in vivo* gene editing program to lower Lp(a) for the treatment of ASCVD. Verve received a milestone payment from Lilly in conjunction with the nomination of VERVE-301 as the development candidate.

Corporate Updates

- Verve today announced that Frederick T. Fiedorek, M.D., will retire from his position as Chief Medical Officer, effective February 28, 2025. Dr. Fiedorek will continue with the company in an advisory capacity. Scott Vafai, M.D., Senior Vice President, Clinical Development, will assume responsibility for clinical development, clinical operations, and medical affairs. Dr. Vafai joined Verve in 2020 and has played a pivotal role in establishing and executing the clinical development plan for Verve's PCSK9 program.
- Verve today announced plans to independently progress a novel, *in vivo* gene editing program for liver disease, previously part of a collaboration agreement with Vertex Pharmaceuticals. Vertex notified the company of its decision to terminate the research collaboration due to changing priorities within its development portfolio. Verve has now regained all rights to develop this nonclinical-stage program.

Upcoming Investor Events

- Barclays Annual Global Healthcare Conference, March 11 at 2 PM ET, Miami, FL
- Jefferies Biotech on the Beach Summit, March 12, 1x1 meetings only, Miami, FL

Upcoming Medical Meeting Presentations

- European Atherosclerosis Society (EAS) Congress, May 4-7, Glasgow, UK

Fourth Quarter and Full Year 2024 Financial Results

Cash Position: Cash, cash equivalents, and marketable securities were $524.3 million as of December 31, 2024, compared with $624.0 million as of December 31, 2023. Verve expects its capital position, including the milestone payment received from Lilly in February 2025, to be sufficient to fund its operations into mid-2027.

Collaboration Revenue: Collaboration revenue was $13.1 million for the fourth quarter of 2024, and $32.3 million for the year ended December 31, 2024, compared to $5.1 million for the fourth quarter of 2023 and $11.8 million for the year ended December 31, 2023. The increase was primarily due to an increase in research services performed under the company's collaboration agreements.

Research & Development (R&D) Expenses: R&D expenses were $55.0 million for the fourth quarter of 2024, and $204.3 million for the year ended December 31, 2024, compared to $46.8 million for the fourth quarter of 2023, and $184.9 million for the year ended December 31, 2023. Stock-based compensation expense included in R&D expenses was $5.3 million and $22.9 million for the fourth quarter and year ended December 31, 2024, respectively, and $4.9 million and $19.1 million for the fourth quarter and year ended December 31, 2023, respectively.

General & Administrative (G&A) Expenses: G&A expenses were $14.1 million for the fourth quarter of 2024 and $56.6 million for the year ended December 31, 2024, compared to $12.3 million for the fourth quarter of 2023, and $49.9 million for the year ended December 31, 2023. Stock-based compensation expense included in G&A expenses was $5.1 million and $20.4 million for the fourth quarter and year ended December 31, 2024, respectively, and $4.4 million and $16.0 million for the fourth quarter and year ended December 31, 2023, respectively.

Net Loss: Net loss was $50.0 million, or $0.58 basic and diluted net loss per share, for the fourth quarter of 2024 and $198.7 million, or $2.35 basic and diluted net loss per share, for the year ended December 31, 2024, compared to $48.4 million, or $0.69 basic and diluted net loss per share, for the fourth quarter of 2023 and $200.1 million, or $3.12 basic and diluted net loss per share, for the year ended December 31, 2023.

About Verve Therapeutics
Verve Therapeutics, Inc. (Nasdaq: VERV) is a clinical-stage company developing a new class of genetic medicines for cardiovascular disease with the potential to transform treatment from chronic therapies to single-course gene editing medicines. The company's lead programs – VERVE-102, VERVE-201, and VERVE-301 – target the three cholesterol drivers of atherosclerosis: LDL-C, remnant cholesterol, and Lp(a). VERVE-102 is designed to permanently turn off the *PCSK9* gene in the liver and is being developed initially for heterozygous familial hypercholesterolemia (HeFH) and ultimately to treat patients with established atherosclerotic cardiovascular disease (ASCVD) who continue to be impacted by high LDL-C levels. VERVE- 201 is designed to permanently turn off the *ANGPTL3* gene in the liver and is initially being developed for refractory hypercholesterolemia, where patients still have high LDL-C despite treatment with maximally tolerated standard of care therapies, and homozygous familial hypercholesterolemia (HoFH). VERVE-301 is designed to permanently turn off the *LPA* gene to reduce Lp(a) levels. Lp(a) is a genetically validated, independent risk factor for ASCVD, ischemic stroke, thrombosis, and aortic stenosis. For more information, please visit www.VerveTx.com.

Cautionary Note Regarding Forward Looking Statements
This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 that involve substantial risks and uncertainties, including statements regarding the company's ongoing Heart-2 clinical trial and Pulse-1 clinical trial; the timing and availability of data for the Heart-2 trial and timing for initiation of the Phase 2 clinical trial for the PCSK9 program; the timing of updates for the PCSK9 and ANGPTL3 programs; the timing of Lilly's opt-in decision for the PCSK9 program; the company's research and development plans; the potential advantages and therapeutic potential of the company's programs; and the period over which the company believes that its cash, cash equivalents and marketable securities will be sufficient to fund its operating expenses. All statements, other than statements of historical facts, contained in this press

release, including statements regarding the company's strategy, future operations, future financial position, prospects, plans and objectives of management, are forward-looking statements. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "will," "would" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in, or implied by, such forward-looking statements. These risks and uncertainties include, but are not limited to, risks associated with the company's limited operating history; the company's ability to timely submit and receive approvals of regulatory applications for its product candidates; advance its product candidates in preclinical studies and clinical trials; initiate, enroll and complete its ongoing and future clinical trials on the timeline expected or at all; correctly estimate the potential patient population and/or market for the company's product candidates; replicate in clinical trials positive results found in preclinical studies and/or earlier-stage clinical trials of VERVE-101, VERVE-102, and VERVE-201; advance the development of its product candidates under the timelines it anticipates in current and future clinical trials; obtain, maintain or protect intellectual property rights related to its product candidates; manage expenses; and raise the substantial additional capital needed to achieve its business objectives. For a discussion of other risks and uncertainties, and other important factors, any of which could cause the company's actual results to differ from those contained in the forward-looking statements, see the "Risk Factors" section, as well as discussions of potential risks, uncertainties and other important factors, in the company's most recent filings with the Securities and Exchange Commission and in other filings that the company makes with the Securities and Exchange Commission in the future. In addition, the forward-looking statements included in this press release represent the company's views as of the date hereof and should not be relied upon as representing the company's views as of any date subsequent to the date hereof. The company anticipates that subsequent events and developments will cause the company's views to change. However, while the company may elect to update these forward-looking statements at some point in the future, the company specifically disclaims any obligation to do so.

Investor Contact
Jen Robinson
Verve Therapeutics, Inc.
jrobinson@vervetx.com

Media Contact
Ashlea Kosikowski
1AB
ashlea@1abmedia.com

Verve Therapeutics, Inc.
Select Condensed Consolidated Financial Information
(in thousands, except share and per share amounts)
(unaudited)

Condensed consolidated statements of operations	Three months ended December 31, 2024	Three months ended December 31, 2023	Year ended December 31, 2024	Year ended December 31, 2023
Collaboration revenue	$ 13,080	$ 5,143	$ 32,332	$ 11,758
Operating expenses:				
Research and development	55,048	46,811	204,347	184,946
General and administrative	14,099	12,281	56,645	49,936
Total operating expenses	69,147	59,092	260,992	234,882
Loss from operations	(56,067)	(53,949)	(228,660)	(223,124)
Other income (expense):				
Change in fair value of success payment liability	(205)	(713)	1,538	165
Interest and other income, net	6,310	6,341	28,762	23,166
Total other income, net	6,105	5,628	30,300	23,331
Loss before provision for income taxes	(49,962)	(48,321)	(198,360)	(199,793)
Provision for income taxes	(73)	(32)	(349)	(275)
Net loss	$ (50,035)	$ (48,353)	$ (198,709)	$ (200,068)
Net loss per share, basic and diluted	$ (0.58)	$ (0.69)	$ (2.35)	$ (3.12)
Weighted-average common shares used in net loss per share, basic and diluted	86,874,007	69,671,255	84,722,277	64,175,137

Condensed consolidated balance sheet data	December 31, 2024	December 31, 2023
Cash, cash equivalents and marketable securities	$ 524,281	$ 623,950
Total assets	$ 647,392	$ 752,688
Total liabilities	$ 153,992	$ 153,186
Total stockholders' equity	$ 493,400	$ 599,502